

WHAT IS CLAIMED IS:

1. A method of treating a disease associated with aberrant microsatellite expansion, comprising administering to a mammal in need thereof, a therapeutically effective amount of recombinant adeno-associated virus (rAAV) containing a transgene that encodes a protein selected from the group consisting of MBNL1, MBNL2, MBNL3, and combinations thereof.
2. The method of claim 1, wherein treating comprises ameliorating or eliminating the symptoms of a neuromuscular or neurological condition caused by the aberrant microsatellite expansion.
3. The method of claim 2, wherein the neuromuscular condition is myotonic dystrophy.
4. The method of claim 1, wherein treating comprises reversing the mis-splicing of the Clcn1 skeletal muscle chloride channel.
5. The method of claim 1, wherein treating comprises reversing the mis-splicing of the Amyloid beta (A4) precursor protein (APP).
6. The method of claim 1, wherein treating comprises reversing the mis-splicing of the NMDA receptor NR1 (GRIN1).
7. The method of claim 1, wherein treating comprises reversing the mis-splicing of the Microtubule-associated protein tau (MAPT).
8. The method of claim 1, wherein treating comprises reversing the mis-splicing of the TNNT2 (cTNT) protein.
9. The method of claim 1, wherein the protein is MBNL1.
10. The method of claim 1, wherein the mammal is human.
11. The method of claim 1, wherein the mammal in need of treatment has RNA inclusions in neuronal cells.

12. A pharmaceutical composition comprising a recombinant adeno-associated virus (rAAV) containing a transgene that encodes at least one protein selected from the group consisting of MBNL1, MBNL2, MBNL3, and combinations thereof.

13. The composition of claim 12, wherein the protein is MBNL1.

14. A mouse model for disease associated with aberrant microsatellite expansion, comprising a mouse having a substantial deletion of *Mbnl1* exon 3 (E3) in the mouse genome, wherein said mouse exhibits symptoms typical of a disease associated with aberrant microsatellite expansion in humans.

15. A cell isolated from the mouse of claim 14.

16. The mouse model of claim 14, wherein the symptoms comprise muscle weakness and ocular cataracts.

17. The mouse model of claim 14, wherein the microsatellite repeat expansion disease is caused by a microsatellite expansion in a coding region of DNA.

18. The mouse model of claim 14, wherein the microsatellite repeat expansion disease is caused by a microsatellite expansion in a non-coding region of DNA.

19. The mouse model of claim 14, wherein said mouse exhibits abnormal muscleblind proteins.

20. The mouse model of claim 14, wherein the disease is myotonic dystrophy.

21. The mouse model of claim 14, wherein said mouse has loss of functional ClC-1 protein.

22. The mouse model of claim 14, wherein said mouse has loss of functional Amyloid beta (A4) precursor protein.

23. The mouse model of claim 14, wherein said mouse has loss of functional NMDA receptor NR1.

24. The mouse model of claim 14, wherein said mouse has loss of functional Microtubule-associated protein tau.

25. The mouse model of claim 14, wherein said mouse has loss of functional TNNT2 protein.

26. The mouse model of claim 14, wherein said mouse has loss of functional TNNT3 protein.

5 27. A method of identifying a compound useful in the treatment of disease associated with aberrant
microsatellite expansion, comprising administering a test compound to the mouse of claim 14 and
monitoring said mouse for reduction or inhibition of the symptoms associated with said disease.

10 28. The method of claim 27, further comprising monitoring said mouse for effects other than those
associated with the disease.

29. The method of claim 27, wherein the disease is myotonic dystrophy.